Small molecule tools and scale-up technologies to expand human umbilical cord blood stem and progenitor cells for clinical and research use

Grant Award Details

Small molecule tools and scale-up technologies to expand human umbilical cord blood stem and progenitor cells for clinical and research use

Grant Type: Tools and Technologies III

Grant Number: RT3-07692

Project Objective: To establish a commercially viable protocol to expand umbilical cord blood hematopoietic stem/progenitor cells (UCB HS/PCs) to significantly increase access to UCB HSC transplants. The project builds of lead compounds identified in a small molecule screen done by the applicant in collaboration with GE.

Investigator:

Name: Andrew Leavitt

Institution: University of California, San Francisco

Type: PI

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Name: Michelle Arkin

Institution: University of California, San Francisco

Type: Co-PI

Disease Focus: Blood Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $1,416,600

Status: Active

Progress Reports

Reporting Period: Year 1

View Report

Grant Application Details

Application Title: Small molecule tools and scale-up technologies to expand human umbilical cord blood stem and progenitor cells for clinical and research use

Public Abstract: Tens of thousands of patients need bone marrow transplants (BMT) every year, some for bone marrow (BM) cancers and some for inherited diseases such as sickle cell anemia and thalassemia, but many lack a BM donor. African Americans, Asian Americans, and people of Hispanic descent are more likely than others to lack a stem cell donor.

BMTs provide hematopoietic (blood) stem and progenitor cells (HS/PCs) that replace the patient's diseased BM with healthy BM. The new BM provides all the circulating blood cells throughout life.

Many BMTs use HS/PCs that do not come from the BM. One such 'other' source is umbilical cord blood (UCB). UCB HS/PCs have many advantages over other HS/PC sources (i.e., BM or peripheral blood). For example, we can easily obtain UCB HS/PCs without any risk to the donor, and we can keep the cells stored in freezers to be available when a patient needs them. However, most UCB samples contain too few HS/PCs to be used to treat people.

Expanding the number of HS/PCs in UCB samples will increase the number of clinically usable UCB samples, offering new hope for thousands of patients who currently lack a donor. We previously screened >120,000 compounds for their ability to expand UCB HS/PCS, and identified a short list of lead candidates. This grant will fund the next step in our effort to develop a novel, clinically-useful UCB HS/PC expansion protocol. Successful completion of this proposal will result in life-saving treatment for thousands of patients.

Statement of Benefit to California: Our proposal seeks to establish a novel method to expand umbilical cord blood hematopoietic stem/progenitor cells (HS/PCs) to make bone marrow transplants (BMTs) available to thousands of patients who currently lack a stem cell donor. The benefits to California are wide-ranging:
• Grow California's skilled workforce and create jobs: This project will train scientists in stem cell research and technology, and our success will attract more talent from outside California.
• Increase innovation: This proposal is highly translational, with a goal to move rapidly from bench to bedside. However, our research will also provide basic insights into stem cell biology that can be applied by other scientists to help patients more broadly.
• Enhancing the medical treatment of California residents: Compounds that expand UBC HS/PCs have the potential to improve clinical benefit and reduce health care costs by increasing the success rate of stem-cell transplants. Given California's diverse ethnic population, we have many patients who need a BMT yet lack a donor, so our residents will directly benefit from our success.
• Attracting venture capital and commercialization: We aim to develop technology that will be highly attractive to the biotechnology industry. We have identified GE as a partner to commercialize our reagents and processes. Furthermore, commercially viable compounds will attract venture capital to fund cell therapies and create new biotech jobs for the California economy.

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